

### **Diabetes Fact Sheet**

CIRM funds many projects seeking to better understand diabetes and to translate those discoveries into new therapies.

### Description

Diabetes comes in two forms: type 1 (also known as juvenile) and type 2 (also known as adult). As many as 3 million people in the U.S. have type 1 diabetes, which is the form primarily being targeted by stem cell research.

Type 1 diabetes is an autoimmune disorder where the body's own immune system destroys the cells in the pancreas that make insulin. Insulin normally circulates in the bloodstream after a meal and allows cells of the body to take up sugar and use it for food. Without insulin, cells starve and the sugar builds up in the bloodstream where it can damage the kidneys, blood vessels and retina.

Any potential cure for type 1 diabetes requires replacing the lost insulin-producing cells of the pancreas. Currently, the only cells that can be used for such a transplant come from donated organs, which are in short supply. Such insulin-producing cell transplants are also risky because the cells can be rejected by the recipient's body if they don't receive immune suppressing drugs.

To solve the first problem, groups of CIRM-funded researchers have developed methods to make replacement insulin-producing cells derived from human embryonic stem cells, which can be grown in large amounts. Implanted into mice and rats these cells are able to regulate blood sugar.

To get around the problem of rejection, CIRM-funded teams have placed cells in a device that implants under the skin and shields the cells from the immune system. Other groups are studying how to regulate the immune system to make stem cell-derived transplants safer.

### **Clinical Stage Programs**

### Caladrius Biosciences

Caladrius is targeting the immune system as an alternative strategy for treating patients with type 1 diabetes. This disease causes the immune system to destroy the insulin-producing cells of the pancreas. The team is developing a stem cell-based therapy using the patient's own cells. They will take cells, called regulatory T cells (Tregs), from the patient's own immune system, expand the number of those cells in the lab and enhance them to make them more effective at preventing the autoimmune attack on the insulin-producing cells.

- Read more about the project
- Read more about the clinical trial

### ViaCyte

The team has developed a way of maturing embryonic stem cells into an early form of the pancreatic cells that produce insulin. They then insert those cells into a permeable device that can be implanted under the skin. Inside the device, the cells mature into insulin-producing cells and the hope is that they will help maintain blood sugar levels at a healthy level. The team is currently testing the safety and efficacy of this device in a Phase 1 clinical trial.

- Read more about this project
- Read more about the clinical trial

# Progress and Promise in Diabetes Research

# **CIRM Grants Targeting Diabetes**

Researcher name	Institution	Grant Title	Grant Type	Approved funds
Tejal Desai	University of California, San Francisco	Thin Film Encapsulation Devices for Human Stem Cell derived Insulin Producing Cells	Quest - Discovery Stage Research Projects	\$1,092,063
Yang Xu	University of California, San Diego	Developing induced pluripotent stem cells into human therapeutics and disease models	Early Translational I	\$5,165,028
Julie Sneddon	University of California, San Francisco	Designing a cellular niche for transplantation of human embryonic stem cell-derived beta cells	Quest - Discovery Stage Research Projects	\$2,006,076
Olivia Kelly	ViaCyte, Inc.	Methods for detection and elimination of residual human embryonic stem cells in a differentiated cell product	Early Translational I	\$5,405,397
Fouad Kandeel	City of Hope	2009 Rachmiel Levine Diabetes and Obesity Symposium: Advances in Diabetes Biology, Immunology and Cell Therapy	Conference	\$15,000
Evert Kroon	ViaCyte, Inc.	Development of the Theracyte Cellular Encapsulation System for Delivery of human ES Cell-derived Pancreatic Islets and Progenitors.	Tools and Technologies I	\$827,072
Fouad Kandeel	City of Hope	2013 Rachmiel Diabetes and Obesity Levine Symposium - Advances in Diabetes Research	Conference	\$27,750
Jeffrey Bluestone	University of California, San Francisco	Stem cell tolerance through the use of engineered antigen-specific regulatory T cells	Transplantation Immunology	\$1,152,768
Fouad Kandeel	City of Hope	2011 Rachmiel Levine Diabetes and Obesity Symposium: Advances in Diabetes Research	Conference	\$15,000
Charles King	University of California, San Diego	Biological relevance of microRNAs in hESC differentiation to endocrine pancreas	Basic Biology III	\$1,313,649
Fouad Kandeel	City of Hope	2012 Rachmiel Levine Diabetes and Obesity Symposium: Advances in Diabetes Research	Conference	\$15,000
Maike Sander	University of California, San Diego	Deciphering transcriptional control of pancreatic beta- cell maturation in vitro	Basic Biology IV	\$1,258,560
Richard Jove	City of Hope	Innovation and Translational Stem Cell Therapy for Diabetes and Neurological Diseases: Paving the way for real life solutions	Conference	\$14,878

Howard Foyt  Douglas Losordo  Mark Anderson  Esther Latres	ViaCyte, Inc.  Caladrius Biosciences, Inc.  University of California, San Francisco	Preclinical and clinical testing of a stem cell-based combination product for insulin-dependent diabetes  Phase 2 Safety and Efficacy Study of CLBS03 Autologous T-Regulatory Cells in Adolescents with Recent Onset Type 1 Diabetes Mellitus  Generation of a functional thymus to induce immune	Strategic Partnership I Clinical Trial Stage Projects	\$9,475,070 \$12,211,255
Losordo  Mark  Anderson  Esther	Biosciences, Inc.  University of California, San	T-Regulatory Cells in Adolescents with Recent Onset Type 1 Diabetes Mellitus  Generation of a functional thymus to induce immune	_	\$12,211,255
Anderson Esther	California, San	·		
		tolerance to stem cell derivatives	Basic Biology V	\$1,191,000
	JDRF	JDRF Encapsulation Consortium Fall 2017 Meeting	Conference II	\$42,425
David Tirrell	California Institute of Technology	Engineered matrices for control of lineage commitment in human pancreatic stem cells	Basic Biology V	\$526,896
Howard Foyt	ViaCyte, Inc.	Clinical trial of directly vascularized islet cell replacement therapy for high-risk type 1 diabetes	Clinical Trial Stage Projects	\$19,752,463
Jeffrey Bluestone	University of California, San Francisco	A CIRM Disease Team for the Treatment and Cure of Diabetes	Disease Team Planning	\$55,000
Howard Foyt	ViaCyte, Inc.	Clinical Development of a Cell Therapy for Diabetes	Accelerated Development Pathway I	\$8,783,852
Howard Foyt	ViaCyte, Inc.	Clinical trial of directly vascularized islet cell replacement therapy for high-risk type 1 diabetes	Clinical Trial Stage Projects	\$19,752,463
Catriona Jamieson	University of California, San Diego	Alpha Stem Cell Clinic for the Development of Regenerative Therapies	Alpha Stem Cell Clinics	\$7,999,137
Mark Walters	Children's Hospital of Oakland Research Institute	University of California, San Francisco (UCSF) CIRM Alpha Stem Cell Clinic	Alpha Stem Cell Clinics Network Expansion	\$7.999.999
Roslyn Isseroff	University of California, Davis	Scaffold for dermal regeneration containing pre- conditioned mesenchymal stem cells to heal chronic diabetic wounds	Preclinical Development Awards	\$4,620,144
Felicia Pagliuca	Semma Therapeutics	Personalized Cell Therapy for Diabetes	Therapeutic Translational Research Projects	\$555,890
Senta Georgia	Children's Hospital of Los Angeles	Developing a personalized approach to beta cell replacement for patients with a genetic form of diabetes	Inception - Discovery Stage Research Projects	\$180,000
Allan Robins	ViaCyte, Inc.	Cell Therapy for Diabetes	Disease Team Research I	\$22,999,933
Kevin D'Amour	ViaCyte, Inc.	Stem cell-derived islet cell replacement therapy with immunosuppression for high-risk type 1 diabetes	Late Stage Preclinical Projects	\$3,544,721

Didier Stainier	University of California, San Francisco	Endodermal differentiation of human ES cells	SEED Grant	\$611,027	
					Total: \$138,609,516.00

### **CIRM Diabetes Videos**



Diabetes: Progress toward stem cell therapies, a Live Google Hangout



Ron Piran, Sanford-Burnham - CIRM Stem Cell #SciencePitch



Eugene Brandon, Viacyte, Inc. - CIRM Stem Cell #SciencePitch



Diabetes: Progress and Promise in Stem Cell Research



Diabetes: Advancing Stem Cell Therapies - 2011 CIRM Grantee Meeting



Alan Lewis Talks About Getting an Embryonic Stem Cell-Based Therapy to Patients



Spotlight on Type 1 Diabetes: Welcoming Remarks



Spotlight on Type 1 Diabetes: Seminar by Ed Baetge, Ph.D.



Spotlight on Type 1 Diabetes: Seminar by Peter Butler, M.D.



Spotlight on Type 1 Diabetes: Seminar by Joelle Johnson



Stem Cell Based Diabetes Treatment: Alan Lewis - CIRM Science Writer's Seminar

### **News and Information**

- CIRMResearch blog entries on type 1 diabetes research progress
- CIRM Loan to Boost Diabetes Research (San Diego Business Journal)
- Diabetes Health Guide (New York Times)
- San Diego company studies stem cell implant as a Type 1 diabetes treatment (Los Angeles Times)

## Resources

- NIH: Diabetes Information
- CDC: Diabetes Resources
- Find a clinical trial near you: NIH Clinical Trials database
- Juvenile Diabetes Research Foundation
- American Diabetes Association
- Stem Cell Netword diabetes page
- Family Caregiver Alliance
- National Family Caregivers Association

### Find Out More:

 $\textbf{Source URL:} \ https://www.cirm.ca.gov/our-progress/disease-information/diabetes-fact-sheet$